DOI:
10.1002/ppul.24145

Document Version
Peer reviewed version

Title: Respiratory health service utilization of children with neuromuscular disease

Authors

Louise Rose, PhD

Sunnybrook Health Sciences Centre and Sunnybrook Research Institute

Florence Nightingale Faculty of Nursing, Midwifery, and Palliative Care, Kings College London

Lawrence S. Bloomberg Faculty of Nursing and Faculty of Medicine, University of Toronto

Institute of Clinical Evaluative Sciences

Douglas McKim, MD

The Ottawa Hospital Respiratory Rehabilitation and The Ottawa Hospital Sleep Centre

Ottawa Hospital Research Institute

University of Ottawa

David Leasa MD

Department of Medicine, Divisions of Critical Care and Respirology London Health Sciences Centre

Western University

Mika Nonoyama PhD

University of Ontario Institute of Technology

Child Health Evaluative Sciences & Respiratory Therapy, SickKids

Rehabilitation Sciences & Physical Therapy, University of Toronto

Anu Tandon MD

Sunnybrook Health Sciences Centre

University of Toronto

Yu Qing Bai MSc

Institute of Health Policy, Management and Evaluation, University of Toronto

Roger Goldstein MBChB

West Park Healthcare Centre

Faculty of Medicine, University of Toronto

Reshma Amin, MD

Division of Respiratory Medicine, SickKids

SickKids Research Institute

University of Toronto

Sherri Katz, MDCM

Division of Respirology, Children's Hospital of Eastern Ontario

Children's Hospital of Eastern Ontario Research Institute

University of Ottawa

Andrea Gershon MD

Sunnybrook Health Sciences Centre

Sunnybrook Research Institute and the Institute of Clinical Evaluative Sciences,

Department of Medicine, University of Toronto

Institution: This study was conducted at the University of Toronto

Funding source: The study was funded by a Respiratory Care grant from Muscular Dystrophy Canada. Louise Rose holds a Canadian Institutes of Health Research (CIHR) New Investigators Award. Andrea Gershon holds a CIHR Foundation Grant and received funding from a Physicians Services Incorporated

Fellowship for Translational Research while working on this study. Roger Goldstein holds the NSA Chair in Respiratory Rehabilitation Research

Disclaimer: This study was also supported by the Institute for Clinical Evaluative Sciences (ICES), which is funded by an annual grant from the Ontario Ministry of Health and Long-Term Care (MOHLTC). The opinions, results and conclusions reported in this paper are those of the authors and are independent from the funding sources. No endorsement by ICES or the Ontario MOHLTC is intended or should be inferred. Parts of this material are based on data and information compiled and provided by CIHI. However, the analyses, conclusions, opinions and statements expressed herein are those of the author, and not necessarily those of CIHI.

Permission was received to use the Immigration, Refugees and Citizenship Canada (IRCC)'s Permanent Resident Database to characterize immigration status and the Johns Hopkins ACG® system for describing Collapsed Aggregated Diagnosis Groups.

Meetings Presented: These data were presented at the 2017 European Respiratory Society Annual Congress, Milan, 9th-13th Sept.

Corresponding Author

Louise Rose Sunnybrook Health Sciences Centre #D108, Bayview Ave Toronto, ON M4N 3M5

Tel. 416-480-6100 ext7822; louise.rose@utoronto.ca

Keywords: neuromuscular disease; chronic respiratory failure; health care utilization; epidemiology

Short title: Health service utilization of neuromuscular disease

ABSTRACT

Objectives: To quantify health service utilization including assessment, monitoring, and treatment of respiratory complications of children with neuromuscular disease (NMD), identifying practice variation and adherence to guideline recommendations at a population level.

Methods: North American population-based cohort study (2003 to 2015) of children with NMD using hospital diagnostic and physician billing codes within health administrative databases.

Results: We identified 18,163 children with NMD. Mean (SD) age was 7.8 (5.6) years with 40% ≤ 5; 45% were female. Most common diagnoses were cerebral palsy (50%) and spina bifida (16%); 8% had muscular dystrophy. From fiscal years 2003-2014, 15,600 (86%) children went to an emergency department on average 3.5 times every 3 years; 6,575 (36%) for respiratory reasons. 8,788 (48%) were admitted to hospital with 2190 (12%) for respiratory reasons and 2,451 (13%) required intensive care. Respiratory specialist outpatient visits occurred for 2,226 (12%) children on average 6.5 visits every 3 years; 723 (4%) had in-hospital respiratory specialist consultation. Pulmonary function testing was conducted in 3,194 (18%) children on average 2.4 times every 3 years; sleep studies in 1,389 (8%).

Conclusion: In this population-based study of children with NMD, healthcare utilization for respiratory complications was considerable. Frequency of respiratory specialist consultation, monitoring of respiratory function and sleep disordered breathing was variable but on average reflected professional society recommendations. Children with NMD are frequent ED users suggesting a need to improve community and social supports. We did not detect reduced access to respiratory monitoring or specialist consultation in adolescents transitioning to adult services.

INTRODUCTION

Children with neuromuscular disorders (NMD) and their families incur significant direct and indirect healthcare utilization and costs including family caregiving though this varies based on the underlying diagnosis and disease progression [1], though data specific to respiratory complications and across multiple disease variants are lacking. In a US hospital administrative database study of muscular dystrophy (MD), hospitalization rates and duration of hospital stay were substantially higher in the MD group compared with children without MD [2]. Similarly, Ouyang and colleagues [3] reported greater utilization of outpatient and in-patient services and more prescriptions by children and young adults with MD. Given the era of novel therapeutics [4], there is an imperative to generate better understanding of the current state of healthcare utilization for children with NMD to inform policy regarding allocation of services and funding.

Progression of respiratory muscle weakness and the timing of prescription for mechanical ventilation are highly variable among children with NMD. For example, in children with Duchenne muscular dystrophy (DMD) the need for ventilatory support occurs during adolescence [5] but for children with spinal muscular atrophy (SMA) type 1, significant respiratory insufficiency occurs around six months[6] although onset is delayed with children receiving Nusinersen [4]. Respiratory failure resulting from progressive respiratory muscle weakness, reduced lung volumes, impaired cough, aspiration, and pneumonia is the most frequent cause of death in children with NMD [7]. As recommended by professional society guidelines [8-10], optimal assessment and monitoring of respiratory function in the outpatient setting enables early detection of progression of respiratory muscle weakness and prevention of unscheduled emergency department (ED) visits and hospital admissions.

We conducted a population-based cohort study using administrative databases to quantify health service utilization, including services used for assessment, monitoring, and treatment of respiratory complications associated with NMD. We hypothesized that for those children requiring specialized respiratory care, access to such services, and frequency of access would be variable. Secondary objectives were to determine: (1) factors associated with rates of ED presentation or hospital admission; and (2) healthcare utilization before and after transition from pediatric to adult healthcare services.

METHODS

Using health administrative databases for Ontario, Canada held at the Institute for Clinical Evaluative Sciences (ICES), we conducted a retrospective population-based study (2003-2015). Datasets contain anonymized health data for all Ontario residents, the most populous Canadian province, with a population of approximately 13 million [11]. Costs of all medically necessary care are covered by universal public health insurance funded through general taxation.

Data Sources and Population:

We linked the following databases at the level of the individual through unique encoded identifiers: (1) Discharge Abstract Database for demographic and procedural data (including up to 24 comorbidities) including in-hospital death for all hospitalizations; (2) National Ambulatory Care Reporting System for all ED presentations; (3) Ontario Health Insurance Plan database for physician billings including procedures [12]; (4) Assistive Devices Program (ADP) for approvals of assistive devices including ventilators; (5) National Rehabilitation Reporting System for inpatient rehabilitation programs; (6) Continuing Care Reporting System for facility based continuing (residential) care services; and (7) Home Care Database for home care services. To describe immigration status, we used the Immigration, Refugees and Citizenship Canada Permanent Resident Database. This database

contains individual-level demographic data on all immigrants who became permanent residents between 1985 and 2012. Following well-established methods [13], we ascertained neighbourhood income and urban/rural place of residence using postal codes with linkage to Statistics Canada census data. To describe physician specialty, we used the Physician Database that contains information on all physicians practising in Ontario.

For the purposes of describing healthcare utilization, we derived a cohort of children aged 0 to <18 years from April 1st 2003 to March 31st 2008 (5 years) using International Classification of Disease (ICD)-9, ICD-10 codes, and physician billing codes *a priori* identified as specific to NMD. Within this 5 year period, we sought the first instance of ICD codes identified as the 'most responsible' or as a secondary contributing diagnoses in the hospitalization database [14] indicating hospital admission with a NMD diagnosis. For children with NMD identified through ED presentation, we confirmed NMD diagnosis by looking backwards and forwards in time in the hospitalization database (1998-2014) for a NMD-related hospital admission. For those without ED or hospital admission, we reviewed the physician-billing database for NMD-related billing in these years. For children identified as having NMD using the physician billing code 349 (NMD diagnosis non-specific), we again searched the hospital (1998-2014) and ED (2000-2014) databases for a NMD specific ICD codes and examined billings from a neurologist or for an electromyogram to clarify diagnosis.

We grouped ICD and physician billing codes into 10 categories: cerebral palsy [15]; Guillain-Barre [16]; metabolic disorders; multiple sclerosis [17]; MD; myasthenia gravis [18]; NMD diagnosis non-specific (i.e. physician billing code 349); neuropathy; spina bifida; and SMA. For modelling purposes, we further categorized NMD as progressive (Guillain-Barre syndrome; multiple sclerosis; myasthenia gravis; neuropathy; spinal muscular atrophy; metabolic disorders; muscular dystrophy; neuromuscular disorders

(non-specific); and non-progressive (cerebral palsy; spina bifida). We used previously validated case definitions to identify children who also had asthma [19]. To describe co-morbidities, we determined both Collapsed Aggregated Diagnosis Groups (ADG) using the John Hopkin ACG® System Vers 10.0 [20] and calculated Charlson co-morbidity score considering all hospitalizations within 2 years prior to identification of NMD excluding the index hospitalization. To define transition to adult healthcare services, we used the physician speciality database and examined the physician billing database to determine first contact with an adult specialist around ages 18 to 19.

Outcomes

To establish outcomes related to healthcare utilization, we followed the 2003-2008 cohort for a minimum of seven years to 2015. Our primary outcome was quantification of health service utilization for assessment, monitoring, and management of respiratory complications including respiratory related ED presentations and hospital admissions; consultations with a respiratory specialist (in hospital and ambulatory/outpatient clinic); pulmonary function testing (PFT); polysomnography (PSG); access to community respiratory therapists; and use of respiratory therapies in the community including oxygen, mechanical ventilation; and airway clearance devices.

Additional public healthcare utilization data included: all cause ED presentations; all cause hospital admissions and duration of hospital stay; ICU admissions and duration of ICU stay; outpatient clinic visits to specialists other than respirology; acute care services billed by specialists other than pulmonology; rehabilitation; homecare services; long-term care residence. We examined factors associated with all cause ED presentation and hospital admission considering the following a *priori* identified potentially confounding demographic and clinical variables: NMD category [21]; age; sex; neighbourhood income quintile; rural residence; immigration status; approval for a home ventilator; use

of other medical technologies (ICD codes for gastrostomy, ileostomy, colostomy, cystostomy, dialysis, pacemaker functional implants), comorbidities (asthma, myopathy, arrhythmias); and level of comorbidity using ADGs categorized as many ≥ 8 conditions or few 1-7 conditions) [20]. We compared healthcare utilization before and after transition from pediatric to adult healthcare services.

Ethical considerations

We conducted our study according to a pre-specified protocol approved by the research ethics board at Sunnybrook Health Sciences Centre and according to privacy regulations of ICES.

Statistical analyses

We characterized our cohort using descriptive statistics. We used counts, proportion overall, and frequency over three years per child for healthcare utilization as well as mean (standard deviation (SD)), median (interquartile range (IQR)) of episodes of healthcare utilization. We created generalized linear regression models with negative binomial distribution to examine variables associated with ED presentation and hospital admissions controlling for variables described above. We used t tests to compare rates of healthcare utilization before and after transition from pediatric to adult healthcare services. All analyses were conducted in SAS Enterprise Guide 7.1 (SAS Institute Inc., Cary, NC, USA).

RESULTS

Health service utilization for respiratory complications

We identified 18,163 children with NMD from 2003 to 2008. Of these, 357 (2.0%) had the first instance of NMD codes identified through ED presentation, 1,452 (8.0%) through hospitalization, and 16,354 (90.0%) through physician billing. Mean age was 7.8 years, 55% were male, and 87% lived in an urban location. Asthma (26%) was a fairly common comorbidity (Table 1).

From fiscal years 2003-2014, subsequent to identification of NMD diagnosis, 6,575 (36.2%) children with NMD went to the ED for respiratory reasons (median [IQR] length of stay 4.6 [2.3, 10.4]) hours, and 2,190 (12.1%) were admitted to hospital for respiratory reasons (median [IQR] length of stay 5 [2, 15] days). Frequency of outpatient clinic visits to a respiratory specialist was relatively stable (6.8 (28.1) visits in 2003-2005 and 6.5 (25.9) visits in 2012-2014 as were rates of PFTs and PSGs for children undergoing testing. (Table 2). Respiratory related ED visits and hospital admissions were similar for children with progressive and non-progressive disease but utilization of respiratory specialists, PFTs and PSGs by children with progressive disease was nearly double that of those with non-progressive disease. A publicly funded continuous positive airway pressure device was supplied to 48 (0.3%) children, home oxygen to 328 (1.8%), and 442 (2.4%) were supplied a publicly funded home ventilator (invasive or non-invasive). Only 26 (0.1%) children received a home visit from a respiratory therapist in the community.

Other health service utilization

From 2003-2014, subsequent to identification of NMD diagnosis, 15,578 (85.8%) children went to the ED (all cause) with a median [IQR] length of stay of 14 (5.9, 34.2) hours. Hospital admission occurred for 8,761 (48.2%) children with a median [IQR] length of stay of 7 (2, 20) days. ICU admission occurred for 2,446 (13.5%) (median (IQR) length of stay of 3.5 (1.2, 12.1) days. The average number of outpatient clinic visits to pediatric specialists other than pulmonology every 3 years increased from 12.1 (11.9) (2003-2005) to 13.9 (16.8) (2012-2014). Only 1,993 (11.1%) were seen by a neurologist in an outpatient setting on average 3.5 times every 3 years. Of the cohort, 8,597 (47.3%) NMD children received home care services for a median (IQR) of 63 (17, 517) total hours in one year. Most common services were occupational therapy (2,283, 26.6%), physiotherapy (1,676, 19.5%) and case management

(1,326, 15.4%). Admission to a rehabilitation facility occurred for 116 (0.6%) children with a median (IQR) length of stay of 21 (7, 43) days. Admission to a long-term care facility occurred for 78 (0.4%) children.

Factors associated with ED Presentation or Hospital Admission

Our multivariable models indicate that children with diagnoses other than cerebral palsy or spina bifida had more ED visits and hospital admissions (P <.0001) as did the youngest age category (P<.0001) and poorest income quintile (P<.0001). Girls had more ED visits than boys but no difference in the frequency of hospital admissions. There was no association with frequency of hospital admission and home location, immigration status, ventilation requirement, asthma, cardiomyopathy or arrhythmia. Children living in rural locations, Canadian citizens, and those requiring ventilation had more frequent ED visits, as did children with asthma (Table 3).

Health Service Utilization Before and After Transition to Adult Healthcare

Of the 18,163 children that formed our 2003-2008 cohort, 2,451 (13%) transitioned from pediatric to adult healthcare services. In the 3 years before and after transition we found an increase in all-cause ED visits (mean (SD) 3.8 (5.0) to 4.3 (6.4), P=0.02)) and increased length of ED visit (13.4 (24.9) versus 17.8 (35.9) hours, P<.0001). Length of respiratory related ED visits also increased (P=0.003). Conversely, length of all cause hospital admissions reduced from 17.0 (35.9) to 12.4 (28.7) days (P=0.03). There were no differences in frequency of pulmonologist consults, PFTs, or PSGs. We found no differences in the frequency or duration of health service utilization in the one year before and after transition (Table 4).

DISCUSSION

In this retrospective population-based study of children with NMD, we detected a substantial burden of healthcare utilization for respiratory reasons with over one third visiting the ED (42% of all ED visits, on average twice every 3 years, and 12% requiring hospital admission (24% of all hospital admissions) for respiratory related causes. However, only 4% of our cohort was seen by a respiratory specialist in hospital, and 12% was seen by a respiratory specialist in an outpatient setting. Children under respiratory specialist care were seen frequently (average every 6 months), however substantial variation was noted. Only 2.4% children received mechanical ventilation (either invasive or non-invasive). Other healthcare utilization was common among the cohort, particularly ED visits with at least one visit a year on average and some children attending the ED multiple times a year. Following transition from pediatric to adult services, there was no change in utilization of respiratory related healthcare services or hospitalization rates.

In our cohort, although the overall proportion of children receiving specialist respiratory care was relatively low, children with diagnoses other than cerebral palsy and spina bifida had higher rates of respiratory specialist consultation and disease monitoring through PFTs and PSGs. For those children receiving respiratory specialist care, the frequency of outpatient clinic visits and lung function monitoring, was on average consistent with current guideline recommendations [8-10], however as hypothesized we found substantial variability in terms of the frequency of visits and monitoring as demonstrated by the standard deviations reported in Table 2. This is likely attributable to a combination of referral patterns, access, and disease progression, factors difficult to determine from health administrative databases. Current guidelines recommend 6 monthly out-patient follow-up and PFTs annually in children with progressive disease. PSGs were conducted 1.4 times in three years, although again with substantial variability, which is slightly less frequent than the recommended annual assessment [8], and again likely attributable to a combination of referral patterns, access, but also

Canadian pediatric sleep laboratory capacity [22]. Respiratory out-patient follow-up was more frequent than we have previously reported for adults with NMD (average of 4 visits every 3 years) [23], however we found similar frequency of PFTs and PSGs.

Our data indicate children with NMD are frequent users of the ED with only 15% never visiting the ED, and more than one visit each year on average for those children that did. As reported in studies of ED visits for other conditions, younger age was associated with recurrent ED visits [24, 25]. Recurrent ED use may reflect suboptimal access to community healthcare and supports [26], lack of access to specialty care, lack of care integration, lack of respite, and inexperience of general practitioners with this disease [27, 28]. From our data, we can conclude that strategies are needed urgently to improve community and social supports for children with NMD and their families.

This study represents one of the largest cohorts reporting healthcare utilization of adolescents with NMD before and after transition from pediatric to adult healthcare services. The absence of a reduction in respiratory monitoring or specialist consultation suggests no change in access to, or accessibility of, these services. We did find an increase in all-cause, but not respiratory related ED presentations, and an increase in ED visit length likely reflective of prolonged ED wait times for adults in Canada [29]. Cohen and colleagues also noted stable patterns of health care utilization after transfer from pediatric to adult care in a Canadian population-based study of youth with chronic conditions [30]. Our findings differ from studies in other countries of adolescents with complex medical conditions, including those requiring mechanical ventilation, that report increased healthcare drop-out and poor treatment adherence resulting in increased use of acute healthcare services [31-34]. Identification of effective transition models is of particular importance given further increases in life expectancy as more novel therapeutics become available.

Limitations of our study include: potential for misclassification, particularly using physician-billing codes to identify NMD and the inability to further classify the type of NMD in those children identified using physician billing code 349. Oskoui and colleagues reported classification errors using ICD coding to estimate the prevalence of cerebral palsy [35]. Second, limitations of health administrative database coding prevented more disease specific description of healthcare utilisation i.e., Duchenne muscular dystrophy, the most common muscular dystrophy, as well as an estimation of either disease severity or disease progression on an individual patient level or within disease categories. Third, we were unable to determine ambulatory status to further explore compliance with disease progression specific guideline recommendations [9, 10] or stratify results by disease stage. Fourth, we were unable to assess use of airway clearance strategies as these devices were only publicly funded, and therefore detectable in the ADP database, from 2014 onwards.

CONCLUSION

Healthcare utilization for respiratory complications comprised 42% and 24% of all ED visits and hospital admissions.. Frequency of respiratory specialist consultation, monitoring of respiratory function and sleep disordered breathing was variable but on average reflected the recommendations of professional societies. Children with NMD are frequent users of the ED suggesting strategies are needed urgently to improve community and social supports Adolescents experiencing transition from paediatric to adult healthcare services did not experience a reduction in access to respiratory monitoring or specialist consultations.

ACKNOWLEDGEMENTS

We would like to acknowledge Immigration, Refugees and Citizenship Canada for use of their database and the Johns Hopkins ACG® system.

REFERENCES

1. Klug C, Schreiber-Katz O, Thiele S, Schorling E, Zowe J, Reilich P, et al. Disease burden of spinal muscular atrophy in Germany. *Orphanet J Rare Dis*. 2016;11:58.
2. Bell C, Kurosky S, Candrilli S. Muscular dystrophy-related hospitalizations among male pediatric patients in the United States. *Hosp Pract*. 2015;43:180-185.
3. Ouyang L, Grosse S, Kenneson A. Health care utilization and expenditures for children and young adults with muscular dystrophy in a privately insured population. *J Child Neurol*. 2008;23:883-888.
4. Finkel R, Mercuri E, Darras B, Connolly A, Kuntz N, Kirschner J, et al. Nusinersen versus sham control in infantile-onset Spinal Muscular Atrophy. *N Engl J Med*. 2017;377:1723-1732.
5. Schreiber-Katz O, Klug C, Thiele S, Schorling E, Zowe J, Reilich P, et al. Comparative cost of illness analysis and assessment of health care burden of Duchenne and Becker muscular dystrophies in Germany. *Orphanet J Rare Dis*. 2014;18:210.
6. Oskoui M, Ng P, Liben S, Zielinski D. Physician driven variation in the care of children with spinal muscular atrophy type 1. *Pediatr Pulmonol*. 2017;52:662-668.
7. D'Amico A, Mercuri E, Tiziano F, Bertini E. Spinal muscular atrophy. *Orphanet J Rare Dis*. 2011;6:71.
8. Hull J, Aniapravan R, Chan E, Chatwin M, Forton J, Gallagher J, et al. British Thoracic Society guideline for respiratory management of children with neuromuscular weakness. *Thorax*. 2012;67:1-40.
9. Bushby K, Finkel R, Birnkrant D, Case L, Clemens P, Cripe L, et al. Diagnosis and management of Duchenne muscular dystrophy, part 2: implementation of multidisciplinary care. *Lancet Neurol*. 2010;9:177-189.

10. Bushby K, Finkel R, Birnkrant D, Case L, Clemens P, Cripe L, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and pharmacological and psychosocial management. *Lancet Neurol*. 2010;9:77-93.

11. Statistics Canada. 2011 Census profile. http://www12.statcan.gc.ca/census-recensement/index-eng.cfm. Published 2013. Accessed 28th Nov 2013.

12. Herring A, Ginde A, Fahimi J, Alter H, Maselli J, Espinola J, et al. Increasing critical care admissions from U.S. emergency departments, 2001-2009. *Crit Care Med*. 2013;41:1197-1204.

13. Stephenson A, Hux J, Tullis E, Austin P, Corey M, Ray J. Socioeconomic status and risk of hospitalization among individuals with cystic fibrosis in Ontario, Canada. *Pediatr Pulmonol* 2011;46:376–384.

14. Juurlink D, Preyra C, Croxford R, Chong A, Austin P, Tu J, et al. Canadian Institute for Health Information Discharge Abstract Database: A validation study. Toronto: Institute for Clinical Evaluative Sciences; 2006.

15. Ray J, Redelmeier D, Urquia M, Guttmann A, McDonald S, Vermeulen M. Risk of cerebral palsy among the offspring of immigrants. *PLoS One*. 2014;9:e102275.

16. Breiner A, Widdifield J, Katzberg H, Barnett C, Bril V, K T. Epidemiology of myasthenia gravis in Ontario, Canada. *Neuromuscul Disord*. 2016;26:41-46.

17. Widdifield J, Ivers N, Young J, Green D, Jaakkimainen L, Butt D, et al. Development and validation of an administrative data algorithm to estimate the disease burden and epidemiology of multiple sclerosis in Ontario, Canada. *Mult Scler*. 2015;21:1045-1054.

18. Kwong J, Vasa P, Campitelli M, Hawken S, Wilson K, Rosella L, et al. Risk of Guillain-Barré syndrome after seasonal influenza vaccination and influenza health-care encounters: a self-controlled study. *Lancet Infect Dis*. 2013;13:769-776.

19. Gershon A, Wang C, Guan J, Vasilevska-Ristovska J, Cicutto L, To T. Identifying patients with physician-diagnosed asthma in health administrative databases. *Can Respir J*. 2009;16:183-188.

20. Austin P, van Walraven C, Wodchis W, Newman A, Anderson G. Using the Johns Hopkins Aggregated Diagnosis Groups (ADGs) to predict mortality in a general adult population cohort in Ontario, Canada. *Med Care*. 2011;49:932-939.

21. Shneerson J, Simonds A. Noninvasive ventilation for chest wall and neuromuscular disorders. *Eur Respir J*. 2002;20:480-487.

22. Katz S, Witmans M, Barrowman N, Hoey L, Su S, Reddy D, et al. Paediatric sleep resources in Canada: The scope of the problem. *Paediatr Child Health*. 2014;19:367-372.

23. Rose L, McKim D, Leasa D, Nonoyama M, Tandon A, Bai YQ, et al. Canadian incidence, prevalence and healthcare utilization for respiratory complications of adults with neuromuscular disease. *European Respiratory Society International Congress*. Milan: Eur Resp J; 2017.

Neuman M, Alpern E, Hall M, Kharbanda A, Shah S, Freedman S, et al. Characteristics of recurrent utilization in pediatric emergency departments. Pediatrics. 2014;134:e1025-1031.

25. Alpern E, Clark A, Alessandrini E, Gorelick M, Kittick M, Stanley R, et al. Recurrent and high-frequency use of the emergency department by pediatric patients. Acad Emerg Med. 2014;21:365-373.

26. DePiero A, Ochsenschlager D, Chamberlain J. Analysis of pediatric hospitalizations after emergency department release as a quality improvement tool. Ann Emerg Med. 2002;39:159–163.

27. Hobson E, Baird W, Partridge R, Cooper C, Mawson S, Quinn A, et al. The TiM system: developing a novel telehealth service to improve access to specialist care in motor neurone disease using user-centered design. Amyotroph Lateral Scler Frontotemporal Degener. 2018:1-11.

28. Peters M, Fitzpatrick R, Doll H, Playford E, Jenkinson C. Patients' experiences of health and social care in long-term neurological conditions in England: a cross-sectional survey. J Health Serv Res Policy. 2013;18:28-33.

29. Georgio G, Guttmann A, QH D. Emergency department flow measures for adult and pediatric patients in British Columbia and Ontario: A retrospective, repeated cross-sectional study. *J Emerg Med*. 2017;53:418-426.

30. Cohen E, Gandhi S, Toulany A, Moore C, Fu L, Orkin J, et al. Health care use during transfer to adult care among youth with chronic conditions. *Pediatrics*. 2016 137:e20152734.

31. Prestidge C, Romann A, Djurdjev O, Matsuda-Abedini M. Utility and cost of a renal transplant transition clinic. *Pediatr Nephrol*. 2012;27(2):295-302.

32. Gurvitz MZ, Inkelas M, Lee M, Stout K, Escarce J, Chang RK. Changes in hospitalization patterns among patients with congenital heart disease during the transition from adolescence to adulthood. *J Am Coll Cardiol*. 2007;49(8):875-882.

33. Heaton J, Noyes J, Sloper P, Shah R. Families' experiences of caring for technology-dependent children: a temporal perspective. *Health Soc Care Community*. 2005;13:441-450.

34. Noyes J. Comparison of ventilator-dependent child reports of health-related quality of life with parent reports and normative populations. *J Adv Nurs*. 2007;58:1-10.

35. Oskoui M, Ng P, Dorais M, Pigeon N, Koclas L, Lamarre C, et al. Accuracy of administrative claims data for cerebral palsy diagnosis: a retrospective cohort study. *CMAJ Open*. 2017;5:E570-E575.

Table 1 Cohort baseline characteristics

	Cerebral palsy	Guillian Barre	Metabolic Disorders	Multiple sclerosis	Muscular dystrophy	Myasthenia gravis	NMD non-specific	Neuropathy	Spina bifida	SMA
Total	9,107	97	245	837	1,504	878	2,508	48	2,910	29
Age (years)										
mean (SD)	7 (5.3)	9 (5.6)	< 6	11 (5.0)	8 (5.4)	10 (6)	9 (6.0)	12 (4.7)	7 (5.7)	6 (5.6)
Sex										
Female	3,841 (42)	36 (37)	108 (44)	445 (53)	538 (36)	435 (50)	1183 (47)	23 (48)	1469 (50)	16 (55)
Male	5,266 (58)	61 (63)	137 (56)	392 (47)	966 (64)	443 (50)	1325 (53)	25 (52)	1441 (50)	13 (45)
Neighbourhood Income Quintile[a]										
1 (poorest/lowest)	1,975 (22)	10 (10)	63 (26.)	149 (18)	290 (19)	176 (20)	492 (29)	7 (15)	542 (19)	< 6
2	1,785 (20)	25 (26)	49 (20)	151 (18)	311 (21)	188 (21)	472 (19)	11 (23)	533 (18)	6 (21)
3	1,780 (20)	26 (27)	52 (21)	180 (22)	301 (20)	170 (19)	493 (20)	10 (21)	620 (21)	< 6
4	1,857 (20)	21 (22)	42 (17)	178 (21)	313 (21)	193 (22)	522 (21)	< 6	632 (22)	6 (21)
5 (richest/highest)	1,679 (19)	15 (15)	36 (15)	174 (21)	278 (19)	149 (17)	525 (21)	15 (31)	571 (20)	7 (25)
Rural Residence[b]										
No	7,925 (87)	88 (91)	222 (91)	758 (91)	1,266 (84)	759 (86)	2,229 (89)	39 (81)	2,480 (85)	22 (76)
Yes	1,177 (13)	9 (9)	22 (9)	79 (9)	236 (16)	119 (14)	278 (11)	9 (19)	427 (15)	7 (24)
Immigration Status[c]										
Citizen	8,590 (94)	91 (94)	237 (95)	777 (93)	1,406 (93)	812 (92)	2,363 (94)	44 (92)	2,813 (97)	26 (90)
Immigration[d]	383 (4)	< 6	< 6	48 (6)	71 (5)	47 (5)	104 (4)	< 6	73 (3)	< 6
Refugee	133 (1)	-	< 6	12 (1)	27 (2)	18 (2)	39 (2)	-	23 (1)	< 6
Comorbidity										
Asthma	2563 (28)	24 (25)	52 (21)	220 (26)	331 (22)	218 (25)	672 (27)	15 (31)	551 (19)	12 (41)
Charlson Comorbidity Index (CCI)										
0	2726 (75)	84 (93)	201 (87)	200 (88)	427 (89)	211 (90)	824 (79)	37 (88)	1257 (91)	24 (89)
1 (1 to 3)	901 (25)	6 (7)	31 (13)	27 (12)	47 (10)	23 (10)	204 (20)	< 6 (12)	113 (8)	< 6
2 (≥4)	22 (1)	-	-	-	8 (2)	< 6 (0)	10 (1)	-	< 6	-

Note: some percentages may not sum to 100 due to rounding. NMD: neuromuscular disease (other); SMA: spinal muscular atrophy; SD: standard deviation

a Unable to establish for 69 children; b Unable to establish for 12 children; c Unable to establish for 6 children; d Immigrant is defined as resident within Ontario as holding either Canadian permanent residency status or a work, student, or other form of visa; e Charlson comorbidity index calculated for those children admitted to hospital considering all hospitalizations within 2 years prior to the index event (7,397 children).

Table 2 Health service utilization after NMD diagnosis

Health service N = 18,163 children	n (%) of children	Mean (SD) number of visits per three fiscal years per child[a]			
		2003-2005	2006-2008	2009-2011	2012-2014
Respiratory related ED visits	6,575 (36.2)	1.9 (1.8)	1.9 (1.8)	1.8 (1.7)	1.7 (1.5)
Respiratory related hospital admission	2,190 (12.1)	1.7 (1.4)	1.7 (1.4)	1.8 (1.6)	1.7 (1.6)
Respirology outpatient clinic	2,226 (12.3)	6.8 (28.1)	8.6 (32.3)	7.1 (32.3)	6.5 (25.9)
Hospital respirology consult	723 (4.0)	3.4 (5.4)	5.0 (19.7)	4.8 (17.3)	6.0 (17.9)
Pulmonary function tests	3,194 (17.6)	2.1 (3.1)	2.3 (2.6)	2.4 (3.7)	2.4 (2.7)
Sleep studies	1,389 (7.6)	1.6 (1.0)	1.7 (1.1)	1.5 (0.8)	1.4 (0.7)
Outpatient clinic (non-respirology)	18,029 (99.3)	12.2 (12.2)	13.6 (14.7)	14.7 (17.3)	14 (16.8)
Hospital consult (non-respirology)	8,817 (48.5)	5.6 (9.7)	6.3 (13)	6.8 (17.7)	7.3 (15.2)
All cause ED visits	15,600 (85.9)	3.3 (3.9)	3.5 (4.2)	3.6 (4.6)	3.5 (4.9)
All cause hospital admission	8,788 (48.4)	2.3 (2.6)	2.3 (2.6)	2.3 (2.7)	2.1 (2.4)
ICU admission	2,451 (13.5)	1.5 (1.2)	1.5 (1.1)	1.5 (1.2)	1.7 (1.4)

a Calculated for individuals in the cohort requiring these services.

ED: Emergency department; ICU: intensive care unit; SD: standard deviation.

Table 3 Variables associated with the number of hospital admissions and ED visits after NMD diagnosis

	Hospital admissions				ED visits			
Variables	Estimate	95% CI		P value	Estimate	95% CI		P value
Neuromuscular disease (reference = cerebral palsy or spina bifida)								
Other neuromuscular diseases	0.38	0.25	0.52	<.0001	0.10	0.06	0.14	<.0001
Age (reference = age 0-5 years)								
6-10 years	-0.74	-0.91	-0.58	<.0001	-0.32	-0.37	-0.27	<.0001
11-17 years	-0.81	-0.96	-0.66	<.0001	-0.18	-0.22	-0.13	<.0001
Female	0.09	-0.03	0.21	0.15	0.07	0.03	0.11	0.0002
Neighbourhood income quintile (reference = 1 poorest/lowest)								
Income quintile 2	-0.39	-0.58	-0.20	<.0001	-0.13	-0.19	-0.07	<.0001
Income quintile 3	-0.36	-0.55	-0.17	0.0002	-0.21	-0.27	-0.15	<.0001
Income quintile 4	-0.43	-0.62	-0.24	<.0001	-0.28	-0.34	-0.22	<.0001
Income quintile 5	-0.40	-0.60	-0.21	<.0001	-0.30	-0.36	-0.24	<.0001
Rural residence	-0.02	-0.21	0.16	0.81	0.57	0.52	0.62	<.0001
Immigration status (reference = citizen)								
Immigrant	0.12	-0.20	0.44	0.47	-0.40	-0.49	-0.30	<.0001
Refugee	0.43	-0.08	0.93	0.10	-0.22	-0.38	-0.06	0.007
Ventilation status (reference = none)								
Invasive ventilation	0.76	-0.19	1.72	0.12	0.67	0.36	0.98	<.0001
Non-invasive ventilation	-0.02	-0.69	0.66	0.96	0.39	0.19	0.60	0.0001
Use of other medical technology[a]	1.61	0.86	2.37	<.0001	0.35	0.11	0.60	0.005
Comorbidities								
Asthma	0.07	-0.08	0.22	0.35	0.20	0.15	0.24	<.0001
Cardiomyopathy	-0.65	-2.63	1.34	0.52	-0.20	-0.84	0.43	0.53
Arrhythmias	0.70	-0.65	2.05	0.31	0.28	-0.16	0.71	0.21
Collapsed Aggregated Diagnosis Group (reference = few 1-7)								
Many (8-16)	0.86	0.72	0.99	<.0001	0.59	0.55	0.64	<.0001
None	0.66	0.33	0.99	0.0001	0.11	0.00	0.21	0.04

a individuals requiring any of the following: gastrostomy, ileostomy, colostomy, cystostomy, or pacemaker and functional implants.

Table 4 Healthcare utilization before after transition from paediatric to adult healthcare services

N = 2,451	n (%)[a]	Overall prior[b]	Overall after[c]	P value	n (%)	1 year prior	1 year after	P value
Respiratory related ED visits	537 (21.9)	1.6 (1.1)	1.5 (1.0)	0.44	266 (10.9)	1.3 (0.8)	1.3 (0.6)	0.79
Length of ED visit , hrs	-	3.7 (3.5)	5.4 (9.8)	0.003	-	3.5 (3.4)	3.4 (4.4)	0.91
Respiratory related hospital admission	54 (2.2)	1.6 (1.1)	1.4 (0.7)	0.87	19 (0.8)	1.3 (0.8)	1.3 (0.6)	0.78
Length of hospital stay, days	-	8.7 (13.1)	7.3 (9.1)	0.65	-	3.5 (3.4)	3.4 (4.4)	0.91
Respirology outpatient clinic	278 (11.3)	3.2 (8.0)	5.2 (17.9)	0.18	123 (5)	3.9 (10.1)	4.3 (10.1)	0.84
Hospital respirology consult	41 (1.7)	2.0 (2.3)	3.8 (4.7)	0.07	16 (0.7)	1.7 (1.5)	3.6 (4.7)	0.21
Pulmonary function tests	386 (15.7)	2.2 (3.9)	2.3 (3.9)	0.86	189 (7.7)	1.5 (1.4)	1.7 (2.5)	0.65
Sleep studies	113 (4.6)	1.5 (0.7)	1.5 (0.9)	0.74	66 (2.7)	1.4 (0.7)	1.4 (0.7)	0.77
All cause ED visits	2,004 (81.8)	3.8 (5.0)	4.3 (6.4)	0.02	1,343 (54.8)	2.4 (2.4)	2.5 (2.7)	0.63
Length of ED visit, hrs	-	13.4 (24.9)	17.8 (35.9)	<.0001	-	8.5 (12.3)	9.7 (16.6)	0.09
All cause hospital admission	804 (32.8)	2.0 (2.4)	1.8 (1.9)	0.41	386 (15.7)	1.4 (1.1)	1.4 (1.0)	0.88
Length of hospital stay, days	-	17.0 (35.9)	12.4 (28.7)	0.03	-	12.1 (29.8)	9.1 (20.9)	0.22
ICU admission	186 (7.6)	1.7 (1.7)	1.6 (1.3)	0.71	71 (2.9)	1.6 (1.4)	1.3 (0.6)	0.24
Length of ICU admission, days	-	8.1 (13.8)	9.0 (12.2)	0.84	-	9.3 (17.3)	6.5 (8.9)	0.38

All values are medians (interquartile ranges).

a n (%) of individuals that utilized these healthcare services in both the years preceding and the years following transition from paediatric to adult services.

b calculated for the 3 years prior to transition.

c calculated for 3 years after transition.